Assessment of 'established medical science and medical practice' (update 2015)[1]

Summary

Update of assessment framework
With the introduction of the Health Insurance Act (*Zorgverzekeringswet*) and the Long-Term Care Act (*Wet Langdurige zorg*)[2], the Netherlands has succeeded in realising – on the basis of mutual solidarity – supportive social health insurance for all its residents, with a broad basic health care package. The government, which determines what is included in the basic health care package, opted to apply 'established medical science and medical practice' as decision-criterion for all insured care. In other words, only care that is regarded as effective can be included in the package provided under the Health Insurance Act and the Long-Term care Act.

Within the context of the Health Insurance Act and the Long-Term Care Act, our institute plays the role of package supervisor. One aspect of this is that we assess whether care fulfils 'established medical science and medical practice'. In order to promote consistency in these assessments, and also as a way of accounting for our working methods, in 2007 we established and published an assessment framework.[3] Over the years we have evolved and been elaborated upon our working methods. We have incorporated these developments into an updated version of our assessment framework, which is described in this report.

In this report we describe, 'anno today', how we assess the criterion 'established medical science and medical practice' and includes all identified issues that could play a role in our assessments. This does not imply that all issues described are actually involved in every assessment. Which issues play a relevant role depends on each specific file.

The following is a summary of the main outlines of our assessment framework.

Relative effectiveness
When determining 'established medical science and medical practice', we examine whether treatment policy – in view of its favourable and unfavourable consequences – leads to relevant (added) value for the patient in comparison to the standard or usual treatment. In other words: do we feel that the 'net benefit' of the intervention being assessed is a relevant and sufficiently large benefit in comparison to all existing care, and do we have sufficient confidence that this contribution actually takes place? In fact, what we are interested in is the so-called relative – or comparative – effectiveness of an intervention.

We determine the relative effectiveness by making a comparison. In order to make this comparison, we determine, per invention:
- With which intervention will we compare the intervention being assessed?
- What is the target group of the intervention?
- What outcomes do we regard as crucial/important and will we involve in the comparison?
- Do we feel the magnitude of the effect in the comparison (the relative effectiveness) is large enough (in order words, is the magnitude of the effect clinically relevant)?

We base our conclusion on the results of this comparison, and depending on the (un)certainty of the data in the comparison.

Applying the principles of evidence-based medicine
Our working method is that we apply the principles of evidence-based medicine (EBM) for assessing whether care fulfils 'established medical science and medical practice'. EBM was primarily

[1] Zorginstituut Nederland. Beoordeling stand van de wetenschap en praktijk. Diemen, 2015.

[2] The Long-Term Care Act replaced the Exceptional Medical Expenses Act as of 1 January 2015.

[3] CVZ. Assessment of established medical science and medical practice, 2007. Report no. 254. See also the report on the assessment of medical tests. CVZ. Medical tests. Assessment of established medical science and medical practice. Diemen, 2011. Report no. 293.

developed as a tool that helps practicing doctors to make clinical decisions about individual patients, but these principles are currently put to broader use. They are used by professional associations of care-providers when developing guidelines and also for developing policy in the field of public health. In this case EBM is not used for decisions on individual patients, but for recommendations and/or decisions at a population level.

EBM can also be put to good use when assessing whether care fulfils 'established medical science and medical practice'. Once again, this is not about whether care is effective for an individual patient, but whether it is effective for a given indication field. As defined in Dutch legislation, this question should be answered based on 'established medical science and medical practice'. This formulation illustrates that it is not about two separate criteria ('established medical science' and 'established medical practice'), whereby each requires individual assessment and which could potentially lead to conflicting results. The formulation implies that this is a single, integrated, legally defined standard. EBM combines and actually unites the two elements, science and practice.

The core aspect of EBM is that the available evidence – which can range from randomized comparative research to practical experience – is systematically searched, selected and weighted and used in a structured fashion. A key aspect of EBM is that high-quality scientific research carries most weight. Working according to the principles of EBM involves a number of fixed steps: searching for and selecting information, assessing the information found and drawing a conclusion.

The following points are discussed below: integrated assessment of elements of science and practice, appropriate evidence and fixed steps in EBM.

Integrated assessment of elements of science and practice
EBM means that, in addition to scientific insights, the expertise and experience obtained by care-providers and care-users in practice are also involved in the assessment. Our working method has a number of ways in which this is put into practice. For instance, we make use of existing (international) evidence-based guidelines and – where possible – we work along the same lines. Furthermore, when determining the so-called PICOT questions, we first consult the scientific associations of professionals and patients' organisations. Their practical knowledge and experience enables them to provide us with relevant information, for instance about which outcomes/outcome measures should be chosen. The insights and experience obtained by professionals and patients in practice can also – depending on the quality of the evidence found and subject to certain conditions – play a decisive role in determining the final conclusion.

The concept 'established medical science and medical practice' is normative. This means that 'practice' is not: 'health care that individual professionals (usually) provide'. Nor is 'practice' the opinion of individual care-providers (and individual patients) about the value of the intervention. The assessment may eventually include what the professional group as a whole feels is regarded as a representative and/or correct treatment.

Appropriate evidence
The basic principle of EBM is that high-quality scientific research carries most weight. For instance, normally, a randomised comparative study (randomised controlled clinical trial – RCT), that was well-designed and implemented, will give the least risk of a distortion of the effect and thus the greatest degree of certainty about the causal relationship between the intervention and the effects observed. For other study forms, such as for instance, observational research, there is a higher risk of underestimation or overestimation of effectiveness.

However, it is important to realise that an RCT also has its disadvantages and limitations. For instance, an RCT is not always a true reflection of daily practice, due to the use of inclusion and exclusion criteria which may be very strict. Furthermore, an RCT will not always be an appropriate study design (for instance in a case of research about long-term side effects), nor is it always necessary nor feasible. Because of this we have developed a framework on the basis of which we weigh up systematically, and determine what can serve as 'appropriate evidence' in view of the intervention concerned.

Fixed EBM steps
Working according to EBM principle involves a number of fixed steps: searching for and selecting information, assessing the information found and drawing a conclusion.

Step 1: Searching for and selecting information
By using the so-called PICOT questions, we determine the scope of the assessment. PICOT stands for:
- Patient = the relevant patient population;
- Intervention – the intervention being assessed;
- Comparison = the intervention used for comparison purposes (control intervention);
- Outcome = relevant outcomes/outcome measures;
- Time = minimum follow-up period required.

In addition we determine:
- The minimum clinically relevant difference in outcome that is required;
- The so-called 'appropriate evidence profile'.

Based on the PICOT questions we carry out a systematic search for relevant literature in bibliographic databases. In principle, we search only for published and peer-reviewed literature.

Step 2: Assessing and grading the quality of the evidence
We assess the quality of the literature identified and selected (using PICOT) based on a number of aspects: methodological aspects, relevance of the results, generalizability and the research method used (study design). Whenever possible we comply with recent international developments and use the so-called GRADE method. GRADE is the abbreviation of 'Grading of Recommendations, Assessment, Development and Evaluation'. This method helps us to estimate the quality of all the evidence collected, i.e., of the degree of confidence of the estimated effect. An important characteristic of GRADE is that the so-called 'body of evidence' is determined and assessed per outcome measure. In addition, based on well-defined assessment criteria, the quality of evidence can be downgraded (e.g. RCT) or upgraded (e.g. observational studies).

GRADE defines four levels of quality of evidence:
- high quality: a great deal of confidence exists in the estimated effect;
- moderate quality: a moderate confidence exists in the estimated effect;
- low quality: only limited confidence exists in the estimated effect;
- very low quality: little confidence exists in the estimated effect.

Step 3: Determining the final assessment/conclusion
Ultimately, as a final step we decide which conclusion can be formed about the effectiveness based on the literature that has been assessed. As mentioned previously, what we are interested in is the relative effectiveness. This means that we weigh up the following: do we feel that the 'net benefit' of the intervention, in comparison to existing care, is a wanted, relevant benefit, and is sufficiently large. In addition, are we sufficiently confident about the benefit? For our final conclusion about a specific intervention, we include the following arguments/aspects:
- Arguments based on the balance between advantages and disadvantages of the intervention;
- Arguments based on the quality of the scientific evidence;
- Arguments based on the 'appropriate evidence approach';
- Ideas based on grounds/guarantees of professional groups and patients.

We ensure transparent reporting exists for our assessment and for the recommendation based thereupon.

Process of assessment and establishing a recommendation
We have embedded the assessment of 'established medical science and medical practice' in a process that warrants inclusion of the necessary relevant input and that a well-considered recommendation can be adopted. For example, professionals (through their scientific associations), are involved in assessments from the start, as are patients' associations nowadays. The same applies to health insurers. Furthermore, for each assessment we obtain advice from a committee of independent, external experts, the Scientific Advisory Committee (*Wetenschappelijk Adviesraad,*

WAR). This ensures the input of current scientific knowledge and experience in treatment practice in our recommendations.

Specific subjects
This report also discusses a number of specific topics. The following is a short description of these.

Assessing long-term care
There are various reasons why research into the effectiveness of long-term care is not yet taking place. This is hampering the assessment of this type of care against the criterion 'established medical science and medical practice'. We intend to formulate a long-term care specific interpretation of the assessment framework, taking into account the specific characteristics of long-term care. In this report we describe activities we are initiating that (could) help us do this. These include, for instance, redesigning the questionnaire 'appropriate evidence' so it can be used for assessing long-term care. Expectations are that these activities will encourage and promote the implementation of adequate effectiveness research in this sector, which will eventually have a positive effect on the quality of the care provided.

Assessment of medical tests
On 20 January 2011 our institute published the report "Medical tests, assessment of established medical science and medical practice". The basic principles for assessing medical tests recorded therein still apply. When assessing medical tests, our interest goes beyond simply being able to obtain a nice illustration or the correct diagnosis. Naturally, it is very important that a test is reliable, but issuing a positive recommendation on a test must be based on demonstrated positive consequences for health-related outcomes in persons tested. This is why, when evaluating a test, we assess the effectiveness of the entire trajectory, the test-plus-treatment strategy, which is also described as clinical utility. Clinical utility can be determined based on comparative research between the usual ('old') and the proposed ('new') test-plus-treatment strategy. Such direct evidence is often lacking, however. In that case, we construct a model for a comparative analysis in order to determine whether indirect evidence can be helpful in answering the question about clinical utility. We are gradually starting to use the GRADE method in assessing tests too.

Relationship between package management and quality tasks
In addition to tasks in the field of package management, our institute also has tasks in the field of promoting the quality of health care. The term currently used to describe this part of our package of tasks is: Health Care Quality Institute. In this report we discuss briefly the relationship between assessing the 'established medical science and medical practice' of health care within the framework of package management and the assessment of quality standards that our Quality Institute carries out for inclusion in the register. The assessment framework for inclusion in the register does not involve any content-related assessment, as it is limited to assessment of procedural aspects. Therefore, the inclusion of a guideline in the register does not automatically mean that standards have been fulfilled as stipulated by our institute when assessing 'established medical science and medical practice' within the context of package management. A quality standard included in the register describes good care, but inclusion in the register does not automatically mean that the care described in the quality standard is actually insured basic care.

Initiation date and consequences of a recommendation on 'established medical science and medical practice'
Depending on the situation, a recommendation on 'established medical science and medical practice' can have consequences for the rights of insured persons, policy terms, contracting care and the NZa's definitions of provisions/tariffs.